Guidelines for the treatment of hereditary tyrosinaemia (type 1) through the Life Saving Drugs Program

Life Saving Drugs Program

About this program

Through the Life Saving Drugs Program (LSDP), the Australian Government provides subsidised access for eligible patients to expensive life-saving drugs.

Purpose of this document

This document provides guidance for treating physicians with relevant specialist registration who wish to apply for their patients to receive access to subsidised treatment for hereditary tyrosinaemia (type 1) (HT1) through the LSDP.

It describes the criteria for general, initial and ongoing eligibility to access subsidised treatment and the administrative requirements associated with the initial application and annual reapplications.

Treatment of HT1 through the LSDP

Subsidised treatment is available for eligible patients with a confirmed diagnosis of HT1. Subsidised treatment through the LSDP is not available for patients with either type 2 or type 3 hereditary tyrosinaemia.

Drugs currently available for the treatment of HT1 through the LSDP

There is currently one drug subsidised through the LSDP for the treatment of HT1.

The generic name for this drug is nitisinone. There are 2 brands. The trade names are Orfadin® and Nityr™.

The Therapeutic Goods Administration (TGA) registration and Product Information for nitisinone (Orfadin® and Nityr™) can be found on the TGA's website.

Choice of treatment

Treating physicians are to advise the LSDP which brand they prefer to treat their patient.

All patients who are initiated on a brand or transitioned to a different brand through the LSDP are required to remain on the same brand for a period of at least 12 months.

Dosage

The recommended initial dose is 1 mg/kg body weight/day, administered orally. The maximum dosage of nitisinone that is subsidised through the LSDP is 2 mg/kg/day.

Nitisinone must be administered in combination with dietary restriction of tyrosine and phenylalanine.

Prescriptions are to be written in accordance with the Product Information for each brand.

Dose adjustments can be requested at any time by emailing the LSDP.

For Orfadin

Orfadin is available in capsules (2 mg, 5 mg, 10 mg and 20 mg) and oral suspension (4 mg/mL) for paediatric patients. The dose of Orfadin oral suspension should be prescribed in mLs.

For Nityr

Nityr is available in tablets (2 mg, 5 mg and 10 mg). Tablets may be disintegrated in water inside an oral syringe, to be administered as a suspension.

General eligibility requirements

LSDP funding conditions

A patient must meet the LSDP funding conditions in order to be eligible to receive access to Australian Government–subsidised treatment for HT1 through the LSDP.

The current LSDP funding conditions can be found on the program's website.

For HT1, a patient must:

- satisfy the initial and ongoing eligibility criteria as detailed in these guidelines
- participate in the evaluation of effectiveness of the drug by periodic assessment, as directed by these guidelines, or have an acceptable reason not to participate
- not be suffering from any other medical condition, including complications or sequelae of HT1, or being treated by a medicine (for a condition) that may significantly compromise the effectiveness of the drug treatment
- be an Australian citizen or permanent Australian resident who qualifies for Medicare.

In most cases, participation in a clinical trial will not affect a patient's eligibility to access LSDP medicines. However, treating physicians are required to advise the LSDP if their patient is participating in a clinical trial.

Exclusion criteria

Patients are not eligible for subsidised treatment with nitisinone for the treatment of HT1 through the LSDP if they meet any of the following criteria:

- presence of another life threatening or severe disease where the long term prognosis is unlikely to be influenced by therapy
- presence of another medical condition that might reasonably be expected to compromise a response to therapy.

Nitisinone cannot be used following a liver transplant.

Initial eligibility requirements

Diagnosis

The patient must have a confirmed clinical diagnosis of HT1 based on detection of succinylacetone in the urine and/or blood by a laboratory accredited by the National Association of Testing Authorities.

Evidence of diagnostic succinylacetone test in blood and/or urine must be provided to the LSDP as part of an application for subsidised therapy.

For patients currently treated with nitisinone in combination with dietary restriction of phenylalanine and tyrosine, a recent succinylacetone blood and/or urine test and a recent copy of prescription for nitisinone must be provided to the LSDP.

See the initial application form.

Ongoing eligibility requirements

The treating physician must submit the separate reapplication form. to the LSDP by 1 May every year if they wish their patient to continue to receive subsidised treatment through the LSDP.

The reapplication form must demonstrate clinical improvement in the patient or stabilisation of the patient's condition, and evidence to support ongoing eligibility for the treatment of HT1 must be provided.

The treating physician must declare that the patient continues to meet the eligibility criteria to receive subsidised treatment through the LSDP in accordance with these guidelines.

The patient must have previously received LSDP-subsidised therapy with nitisinone for HT1, and the treatment must have been administered in combination with dietary restriction of tyrosine and phenylalanine.

Subsidised treatment may continue unless one or more of the following situations apply:

- failure to comply adequately with treatment or measures
- failure to provide data, copies of test results and the Excel spreadsheet for HT1, evidencing the effectiveness of the therapy
- presentation of a condition listed in the exclusion criteria.

Testing is not funded or subsidised through the LSDP, however some tests may be subsidised through Medicare or available through the treating public hospital.

See the reapplication form for existing patients.

Patients who are applying to recommence treatment following a break should use the reapplication form.